
Induced pluripotent stem cells--opportunities for disease modelling and drug discovery.

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Public Summary:

The ability to generate induced pluripotent stem cells (iPSCs) from patients, and an increasingly refined capacity to differentiate these iPSCs into disease-relevant cell types, promises a new paradigm in drug development - one that positions human disease pathophysiology at the core of preclinical drug discovery. Disease models derived from iPSCs that manifest cellular disease phenotypes have been established for several monogenic diseases, but iPSCs can likewise be used for phenotype-based drug screens in complex diseases for which the underlying genetic mechanism is unknown. Here, we highlight recent advances as well as limitations in the use of iPSC technology for modelling a 'disease in a dish' and for testing compounds against human disease phenotypes in vitro. We discuss how iPSCs are being exploited to illuminate disease pathophysiology, identify novel drug targets and enhance the probability of clinical success of new drugs.

Scientific Abstract:

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